



Access to Care in the U.S.

SHORT TERM (3-5 YEARS)

- Enhance knowledge of SCD among hematologists, primary care providers, ED physicians, mid-level providers, and palliative care specialists.
- Use hub and spoke model or other approaches to improve geographic access to care.
- Implement coordinated care models that incorporate community health workers, ensuring more equal quality of care regardless of where an individual with SCD receives care.
- Fund more implementation research.
- Identify essential health benefits for people with SCD enrolled in Medicaid.
- Enable long-term SCD care and follow-up counselling by leveraging existing newborn screening in states to collect additional data across the lifespan.
- Enhance use of telemedicine to broaden availability of SCD knowledge and expertise.
- Link SCD diagnosis and SCD quality indicators to reimbursement.
- Improve SCD diagnostic codes.
- Improve blood donation and awareness.
- Implement point-of-care diagnostics when available.

LONG TERM (5-10 YEARS)

- Stabilize funding streams and sustainability of programs domestically.
- Seek payment reform to support comprehensive management of pain and other long-term complications.
- Support development and expansion of effective state strategies (“carve-outs”) to enhance public insurance coverage and reimbursement.



Training and Professional Education

SHORT TERM (3-5 YEARS)

- Develop an actionable plan to educate health care providers about best practices in caring for those with SCD, including primary care providers and emergency department physicians.
- Increase the number of providers who are able to care for those with SCD through training and certificate programs.
- Augment pain management expertise through use of best practices and a thorough assessment of reversible conditions known to precipitate pain crises, such as dehydration and infection.
- Develop clinical support tools to ensure quality of care for people with SCD.
- Work with local stakeholders to develop standard-of-care guidelines that apply to specific, low-resource areas domestically.
- Develop management guidelines to accommodate local capabilities.

LONG TERM (5-10 YEARS)

- Create incentives for training and retention in benign hematology.



Research and Clinical Trials

SHORT TERM (3-5 YEARS)

- Develop, support, and sustain hemoglobinopathy clinical trial networks as well as a better infrastructure to better enroll and study people with SCD and share data, including in community settings (need collaboration for sufficient enrollment).
- Investigate combination therapies and new drug-delivery models.
- Develop means to stratify people with SCD according to course of disease, response to therapy, and disease progression.
- Optimize dosing and treatment response predictors for hydroxyurea.
- Optimize SCD transfusion therapy to increase biological understanding and identify principles for accurate blood matching.
- Coordinate fetal hemoglobin studies and related regulation to increase translation to therapies.
- Identify biomarkers for SCD crisis and prognosis that could be used clinically or as clinical trial surrogate outcomes.
- Optimize bone marrow transplantation procedures.
- Develop clinical endpoints that determine benefit to people with SCD globally (these endpoints will likely differ from U.S.-defined endpoints).
- Conduct more research on patient-oriented outcomes such as pain, fatigue, and infertility.
- Increase understanding of pathogenesis and morbidity of SCD genotypes including sickle cell trait.
- Explore relationships with currently engaged regional organizations to facilitate SCD focus, and to conduct clinical trials in specific regions and countries.
- Form a multinational research consortium to support north/south and south/south collaboration with minimum criteria for participation.
- Support study and development of point-of-contact SCD care/monitoring resources.
- Enhance the participation of persons with SCD in setting the research agenda and increasing patient participation in clinical research.

LONG TERM (5-10 YEARS)

- Conduct better longitudinal studies to understand the determinants of prolonged survival and cause of death in people with SCD.
- Continue to study and refine genome-editing therapies with curative potential.
- Improve understanding of vascular system changes and mechanisms of organ damage in SCD.
- Continue to invest in molecular research on SCD pathogenesis.
- Create a validated, SCD-specific functional assessment tool for pain.
- Extend genotype studies to better understand the spectrum of sickle hemoglobinopathies.
- Improve SCD diagnostic methods.



Global Issues

SHORT TERM (3-5 YEARS)

- Establish and/or expand newborn screening and early intervention programs.
- Increase awareness and education of governments and philanthropic groups (extend global awareness beyond individual efforts by establishing a data-driven, solutions-based case describing SCD and outcomes).
- Marry newborn screening programs with treatment (start as pilot studies and identify cultural barriers).
- Work with local stakeholders to develop standard-of-care guidelines that apply to specific, low-resource areas globally.
- Investigate therapeutic modalities that can be implemented worldwide.
- Sponsor a “hemoglobinopathies institute” to foster regional education and training (provide certificate of participation to allow caregivers to remain in SCD clinics as “specialists.”)
- Establish fellowships to bring African and Indian physicians to the United States.

LONG TERM (5-10 YEARS)

- Stabilize funding streams and sustainability of programs globally.
- Develop a structured, thoughtful approach to adult sickle cell care in low-resource settings.
- Enhance use of community-based organizations for care access and advocacy.
- Foster bi-directional training.
- Establish feasibility for bringing high-risk/high-cost curative therapies to low-resource settings.